Development of MyoDys45-55, a gene editing therapy for Duchenne muscular dystrophy

Grant Award Details

Development of MyoDys45-55, a gene editing therapy for Duchenne muscular dystrophy

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-12920

Project Objective: To conduct a well-prepared pre-IND meeting with FDA for a correction of the DMD gene mediated by AAV-delivery of CRISPR/Cas9 as a gene therapy treating Duchenne muscular dystrophy (DMD).

Investigator:

Name:	Courtney Young
Institution:	MyoGene Bio LLC
Type:	PI

Disease Focus: Duchenne Muscular Dystrophy, Skeletal/Smooth Muscle disorders

Human Stem Cell Use: Somatic Cell

Award Value: $3,400,000

Status: Active

Grant Application Details

Application Title: Development of MyoDys45-55, a gene editing therapy for Duchenne muscular dystrophy

Public Abstract:

Translational Candidate

A gene editing therapy for Duchenne muscular dystrophy that permanently removes a hotspot region of patient mutations to restore dystrophin.

Area of Impact

Duchenne muscular dystrophy (DMD), a fatal muscle wasting disease with no cure.

Mechanism of Action

Our therapy uses CRISPR/Cas9 gene editing to permanently remove a hotspot region of DMD patient mutations, which reframes the gene and restores expression of the dystrophin protein. This approach targets the underlying cause of disease by removing out-of-frame mutations that otherwise would result in a lack of dystrophin and Duchenne disease progression. Thus, restoration of dystrophin by our approach is expected to repair and regenerate damaged muscle in DMD.

Unmet Medical Need

Our therapy is for Duchenne muscular dystrophy, a fatal muscle wasting disease with no cure. There are only a few approved therapies, a corticosteroid (standard of care; slightly improves progression) and exon skipping drugs (only for 8-13% of patients; modestly effective with ~1-3% dystrophin).

Project Objective

Pre-IND meeting

Major Proposed Activities

- Assessment of efficacy, pharmacology and safety in rodent and canine models
- Assessment of off-target editing in human cells
- Development of a potency assay and manufacturing partnership

Statement of Benefit to California:

This proposal will advance preclinical development of our gene editing therapy for Duchenne muscular dystrophy. Duchenne is a devastating muscle wasting disease leading to premature death in the 20-30s. It affects ~1 in 5000 boys worldwide, thus there is a fairly high concentration of Duchenne patients in California. There is currently no cure and only a few approved therapies with limited benefit, thus there is a need for disease modifying therapies that aim to restore dystrophin, like ours.
